

Stem Cell Agency Focuses Almost \$38 Million on Colorectal Cancer, a Deadly Childhood Disorder and High Blood Pressure in the Lungs

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Oakland, CA – Colorectal cancer is the second leading cause of cancer death in the U.S., claiming more than 49,000 lives a year. Only around 10 percent of patients survive longer than five years, and for the 40 percent of patients whose cancer has a mutation in the KRAS gene, survival can often be measured in months. Now a new CIRM-funded clinical trial could change that.

That program is one of three new clinical trials that the CIRM governing Board approved today.

"In everything we do there is a real sense of urgency, because lives are at stake," says C. Randal Mills, Ph.D., President and CEO of CIRM. "Our Board's support for these programs highlights how every member of the CIRM team shares that commitment to moving the most promising research out of the lab and into patients as quickly as we can. These are very different projects, but they all share the same goal, accelerating treatments to patients with unmet medical needs."

In the colorectal cancer program, Forty Seven Inc. has been awarded \$10.2 million to use a combination one-two punch to attack the cancer in patients with the KRAS genetic mutation. Cancer stem cells, which help fuel the growth of the disease, have a receptor on their surface called CD47, dubbed by one researcher as the "don't eat me" gene because it tricks the immune system cells responsible for getting rid of tumors into not doing their job. Forty Seven Inc. has an antibody that blocks the CD47 receptor, turning the cancer into a target for both the immune system and an anti-cancer drug called cetuximab, the second element in the one-two punch.

In vivid contrast to colorectal cancer, Adenosine Deaminase Severe Combined Immune Deficiency (ADA-SCID) is a rare form of a rare disease. The condition is also known as "bubble baby" disease because it leaves children with virtually no immune protection from infections and in the past some children were placed in sterile plastic bubble-like environments to protect them. The condition can be fatal within the first years of life or if left untreated.

A team from the University of California, Los Angeles, led by Don Kohn, was awarded \$20 million to take a patient's own blood stem cells, genetically modify them to correct the defect that causes ADA-SCID, and then reinfuse those cells back into the patient. The modified stem cells will then, hopefully, create a new healthy and functioning blood and immune system.

Kohn has already used a similar method to cure 23 SCID children. This latest approach has been approved by the Food and Drug Administration (FDA) for a Phase 2 clinical trial.

The CIRM Board also approved \$7.35 million for a team at Cedars-Sinai Medical Center to test the use of stem cells to treat pulmonary hypertension, caused by high blood pressure in the arteries that go from the heart to the lungs. This is a chronic, life-changing disease that can ultimately lead to heart failure. The team wants to use cells derived from heart stem cells, also known as cardiospheres, to reduce inflammation in the arteries and reduce blood pressure.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov

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